14 December 2015
EMA/739394/2015
Human Medicines Research and Development Support Division

Public summary of the evaluation of a proposed product-specific waiver

DNA, d(P-thio)([2'-0-(2-methoxyethyl)]m5rU-[2'-0-(2-methoxyethyl)]m5rC-[2'-0-(2-methoxyethyl)]m5rU-[2'-0-(2-methoxyethyl)]m5rU-[2'-0-(2-methoxyethyl)]rG-G-T-T-A-m5C-A-T-G-A-A-[2'-0-(2-methoxyethyl)]rA-[2'-0-(2-methoxyethyl)]m5rU-[2'-0-(2-methoxyethyl)]m5rC-[2'-0-(2-methoxyethyl)]m5rC-[2'-0-(2-methoxyethyl)]m5rC), sodium salt for the treatment of transthyretin-related amyloidosis (ATTR amyloidosis)

On 19 June 2015, the Paediatric Committee of the European Medicines Agency agreed a product-specific waiver for the above mentioned medicine. (EMEA-001773-PIP01-15)

What is DNA, d(P-thio)([2'-0-(2-methoxyethyl)]m5rU-[2'-0-(2-methoxyethyl)]m5rC-[2'-0-(2-methoxyethyl)]m5rU-[2'-0-(2-methoxyethyl)]m5rU-[2'-0-(2-methoxyethyl)]rG-G-T-T-A-m5C-A-T-G-A-A-[2'-0-(2-methoxyethyl)]rA-[2'-0-(2-methoxyethyl)]m5rU-[2'-0-(2-methoxyethyl)]m5rC-[2'-0-(2-methoxyethy l)]m5rC-[2'-0-(2-methoxyethyl)]m5rC), sodium salt, and how is it expected to work?

This medicine is expected to prevent the production of abnormal TTR protein and the formation of toxic fibril deposits in multiple tissues including the peripheral nervous system, gastrointestinal tract, and heart resulting in the disease.

This medicine is currently not authorised in the European Union, studies in adults are currently on-going and the medicine is planned to be indicated in adults for the treatment of patients with familial amyloid polyneuropathy.

What was the proposal from the applicant?

The applicant proposed not to do any study in children (from birth to less than 18 years of age), as only a few cases of ATTR amyloidosis have been reported in the paediatric population. Therefore, the applicant requested an exemption (waiver*) from the obligation to study the medicine in any children, in the condition Treatment of transthyretin-related amyloidosis (ATTR amyloidosis).

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Is there a need to treat children affected by transthyretin-related amyloidosis (ATTR amyloidosis)?

Taking into account the proposed indication in adults, and the characteristics of the medicine, the Paediatric Committee considered that this condition predominantly occurs in adults, and very occasionally affects adolescents.

What did the Paediatric Committee conclude on the potential use of this medicine in children?

The Committee agreed with the request of the applicant to be exempted from performing studies in children from birth to less than 18 years, because the Committee concluded that this medicinal product does not seem to have any potential significant benefit as clinical studies are not feasible.

What happens next?

The applicant has now received the EMA Decision (P/0181/2015)* on this medicine. The Decision itself is necessary for the applicant to request in the future a marketing authorisation* for this medicine in adults.

*Definitions:

Applicant	The pharmaceutical company or person proposing the Paediatric Investigation Plan or requesting the Product-Specific Waiver
Children	All children, from birth to the day of the 18th birthday.
Paediatric investigation plan (PIP)	Set of studies and measures, usually including clinical studies in children, to evaluate the benefits and the risks of the use of a medicine in children, for a given disease or condition. A PIP may include "partial" waivers (for example, for younger children) and/or a deferral (see below).
Waiver	An exemption from conducting studies in children, for a given disease or condition. This can be granted for all children (product-specific waiver), or in specific subsets (partial waiver): for example, in boys or in children below a given age.
Deferral	The possibility to request marketing authorisation for the use of the medicine in adults, before completing one or more of the studies /measures included in a PIP. The Paediatric Committee may grant a deferral to avoid a delay in the availability of the medicine for adults.
Opinion	The result of the evaluation by the Paediatric Committee of the European Medicines Agency. The opinion may grant a product-specific waiver, or agree a PIP.
Decision	The legal act issued by the European Medicines Agency, which puts into effect the Opinion of the Paediatric Committee.
Pharmaceutical form	The physical aspect of the medicine (the form in which it is presented), for example: a tablet, capsule, powder, solution for injection, etc. A medicine can have more than one pharmaceutical form.
Placebo	A substance that has no therapeutic effect, used as a control in testing new drugs.
Active control	A medicine with therapeutic effect, used as a control in testing new drugs.
Historical control	A group of patients with the same disease, treated in the past and used in a comparison with the patients treated with the new drug.
Route of administration	How a medicine is given to the patient. For example: for oral use, for intramuscular use, for intravenous use, etc. The same medicine, or the same pharmaceutical form, may be given through more than one route of administration.
Patent	A form of protection of intellectual property rights. If a medicinal product is protected by a patent, the patent holder has the sole right to make, use, and sell the product, for a limited period. In certain circumstances, a patent for a medicinal product may be extended for a variable period by a Supplementary Protection Certificate.
Marketing Authorisation	When a Marketing Authorisation is granted, the pharmaceutical company may start selling the medicine in the relevant country (in the whole European Union, if the procedure was a centralised one).